Title (en)

PRECISE DELETION OF CHROMOSCOMAL SEQUENCESIN

Title (de)

GENAUE DELETION VON CHROMOSOMALEN SEQUENZEN

Title (fr)

SUPPRESSION PRÉCISE DE SÉQUENCES CHROMOSOMIQUESIN

Publication

EP 3289076 A1 20180307 (EN)

Application

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Priority

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- US 2016030460 W 20160502

Abstract (en)

[origin: WO2016179112A1] The present invention provides a method of treating a nucleotide repeat expansion disorder comprising delivering a pair of engineered nucleases, or genes encoding engineered nucleases, to the cells of a patient such that the two nucleases excise the nucleotide repeat responsible for the disease permanently from the genome. The invention provides a general method for treating nucleotide repeat expansion disorders and engineered nucleases suitable for practicing the method. The invention further provides vectors and techniques for delivering engineered nucleases to patient cells.

IPC 8 full level

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